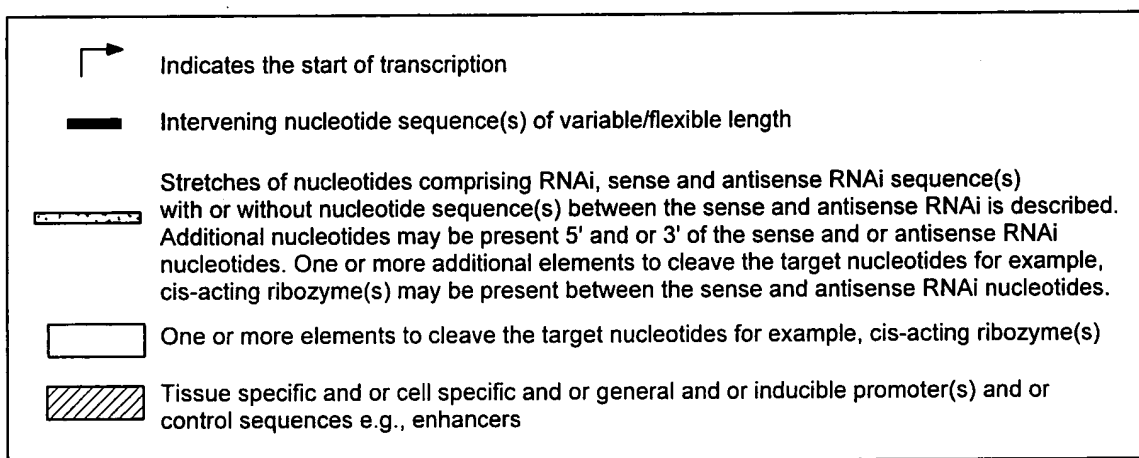
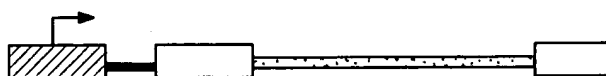


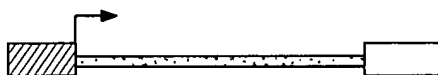
### Design for RNAi expression



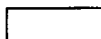
Expression of this cassette may be undertaken via inter alia a viral or non-viral vector, an artificial chromosome and or a stretch of DNA and or RNA

FIG. 1

### Design for RNAi expression



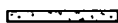
Indicates the start of transcription. RNAi is transcribed close to the start site for transcription



Optional inclusion of none, one or more elements to cleave the target nucleotides for example, cis-acting ribozyme(s) to cleave off for example the polyA tail



Tissue specific and or cell specific and or general and or inducible promoter(s) and or control sequences e.g., enhancers



Stretches of nucleotides comprising RNAi, sense and antisense RNAi sequence(s) with or without nucleotide sequence(s) between the sense and antisense RNAi is described. Additional nucleotides may be present 5' and or 3' of the sense and or antisense RNAi nucleotides. One or more additional elements to cleave the target nucleotides for example, cis-acting ribozyme(s) may be present between the sense and antisense RNAi nucleotides.

FIG. 2

Number	Gene	Reference	5' Sequences driving tissue specific expression
1	Rhodopsin, RHO gene	Gouras et al. (1994) Vis. Neurosci.11: 1227	Photoreceptor specificity can be achieved using various sequences 5' of the TSS of the rhodopsin promoter (from -222 to +70 and -2174 to +70). Expression in both rod and cone photoreceptor cells is observed using these 5' sequences
2	Alpha 1 type I collagen	Kalajuzic et al. (2002) Bone. 31: 654	Expression in osteoblasts can be achieved using, for example, 3.6 kb of the rat type collagen 1A1 promoter
3	Oligodendrocyte myelin glycoprotein	Solly et al. (1997) Neurochem. 68: 1705	657 bases of the 5' murine MOG can drive gene expression of a gene in an oligodendroglial cell line
4	Cone arrestin (CAR)	Zhu et al. (2002) Febs letters 524: 116	215 bases 5' fragment of the CAR gene can drive expression of a gene in retinoblastoma cells
5	Albumin	Postic et al. (1999) J. Biol. Chem. 274: 305	2.3 kilobases 5' of the albumin gene can drive expression of a gene in hepatocytes
6	Neuron specific enolase	Kowalski et al. (2001) Diabetes. 30: 425	1.8 kilobases of the neuron specific enolase drives gene expression in neurons
7	Human GnRH gene	Wolfe et al. Mol. Endo.16: 435	Expression in neurons can be achieved using 992 bases of the human GnRH gene promoter
8	alpha-actin promoter	Frauli et al. (2003) Med. Sci. Monit. 9: Br78	Human skeletal alpha-actin promoter (between -432 and +239) and beta-enolase enhancer (+504 and +637) can direct gene expression in skeletal muscle
9	Smooth muscle myosin heavy chain promoter	Franz et al. (1999) Cardiovasc. Res. 43: 1040	2.3 kilobases of the smooth muscle myosin chain promoter can direct expression to vascular tissue

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Promoter sequence driving tissue specific gene expression

FIG. 3

CAACCACTACCTGAGCACCCAGTTCAAGAGACTGGGTGCTCAGGTAGTGGTTGTC

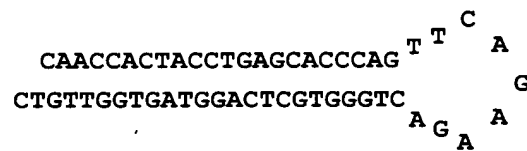


FIG. 4A

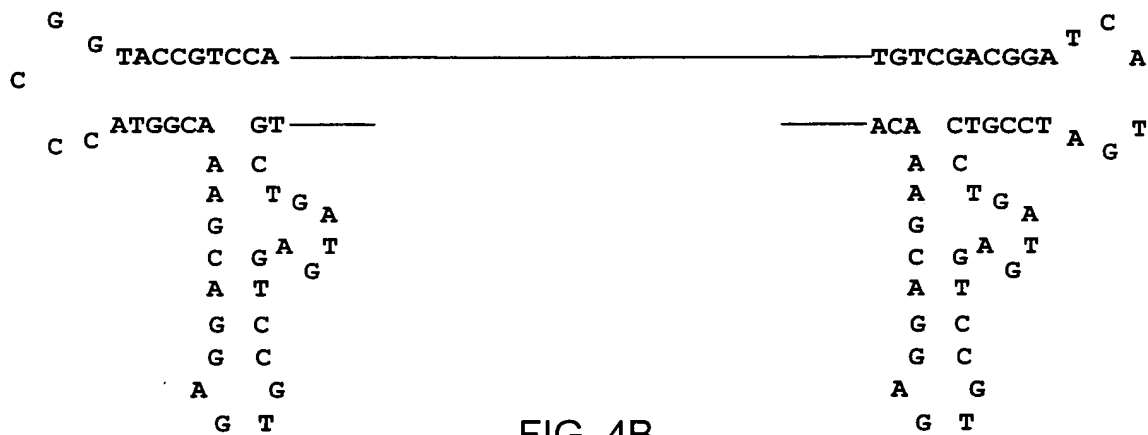


FIG. 4B

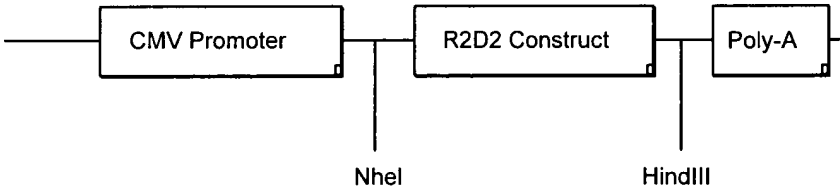


FIG. 4C

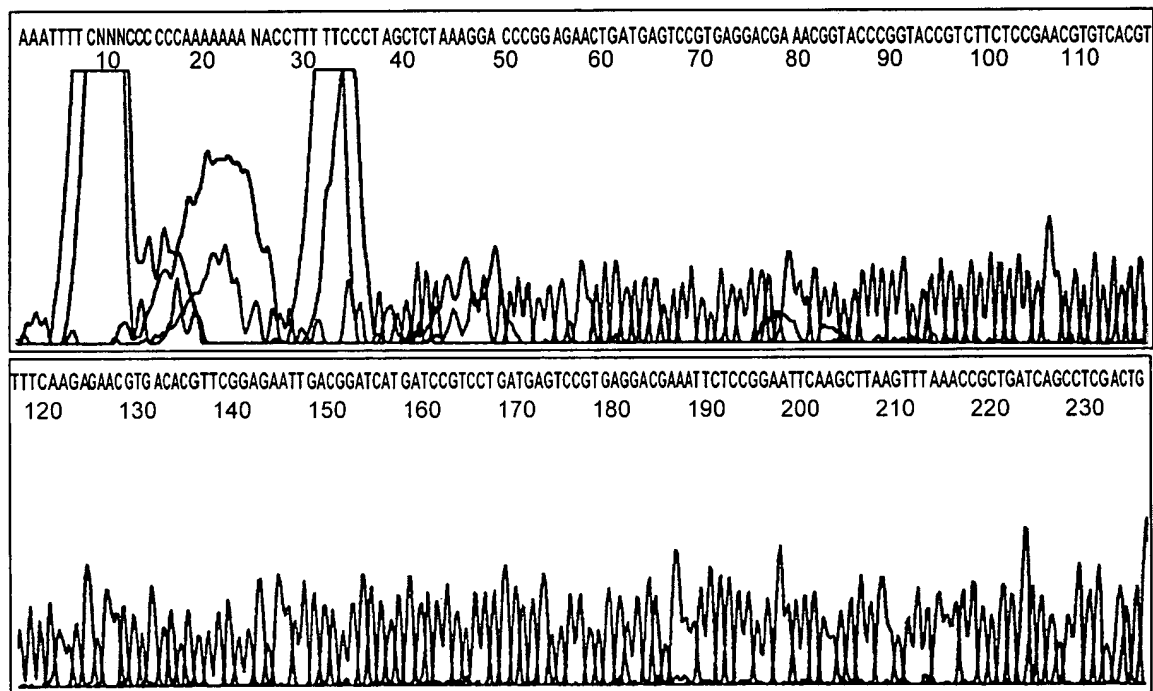


FIG. 4D-1

FIG. 4D-2

FIG. 4D

FIG. 4D-1

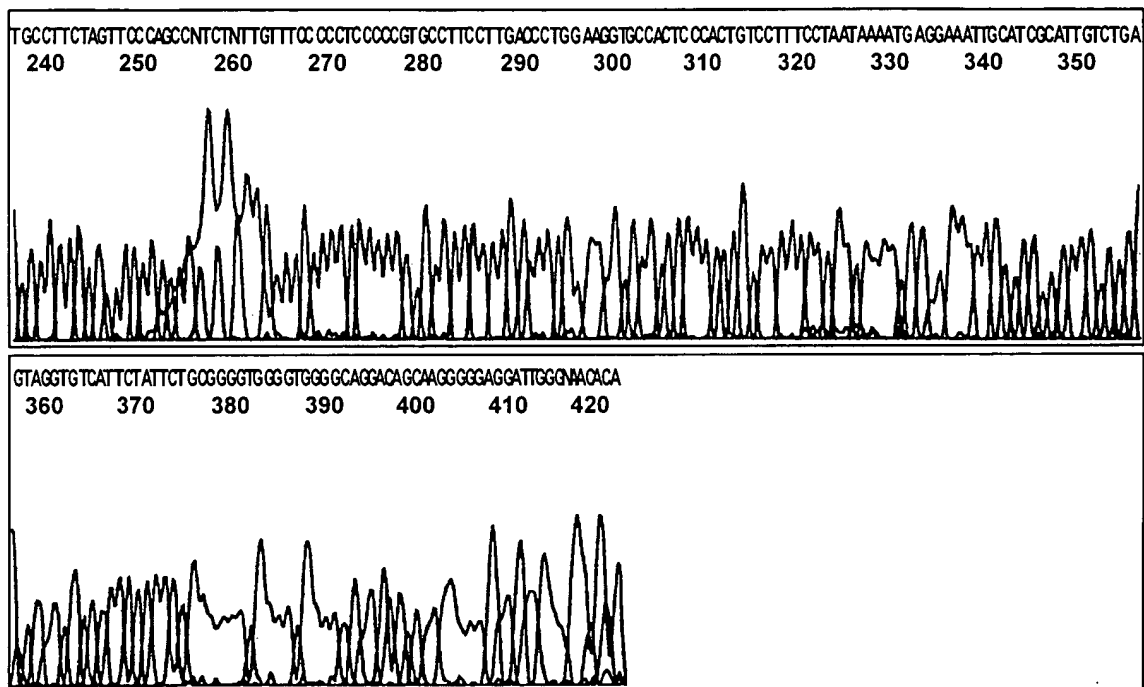


FIG. 4D-2

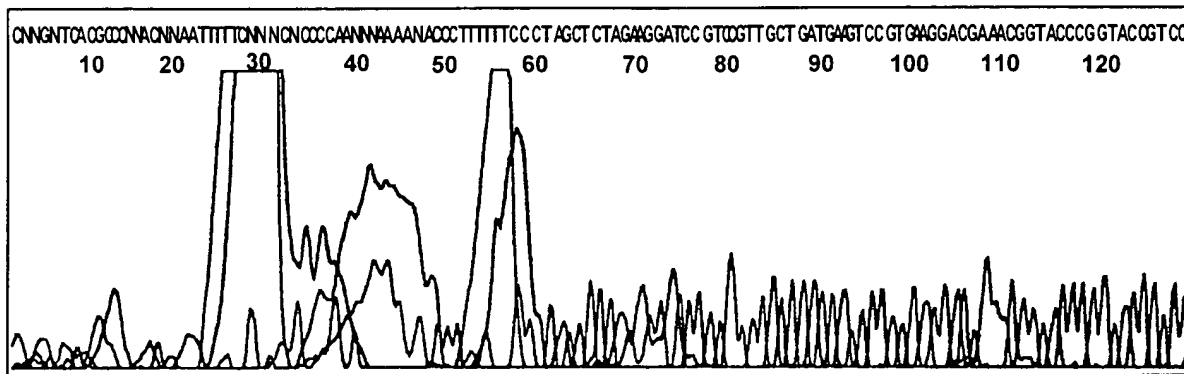


FIG. 4E-1

FIG. 4E-2

FIG. 4E

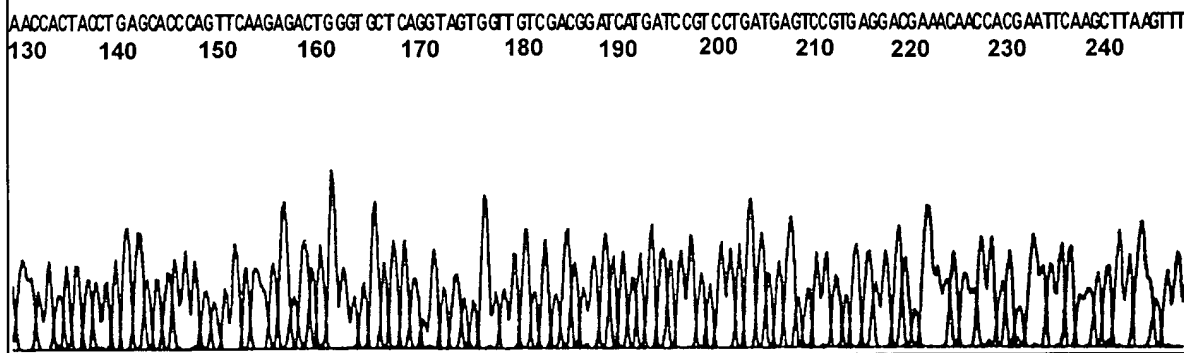


FIG. 4E-1



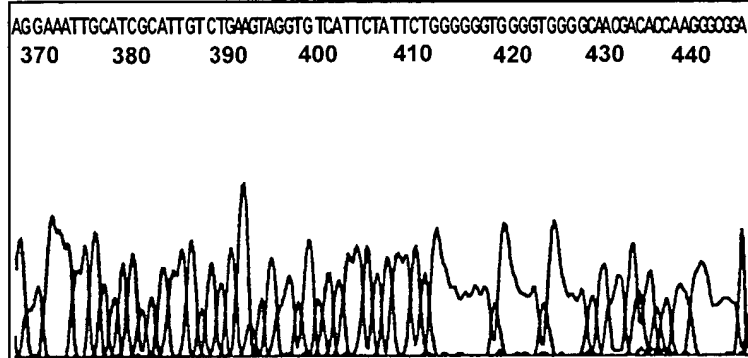
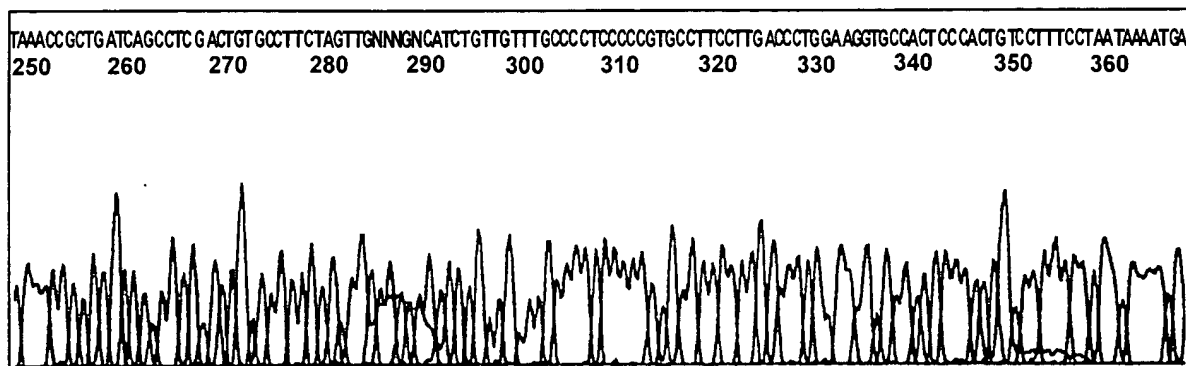


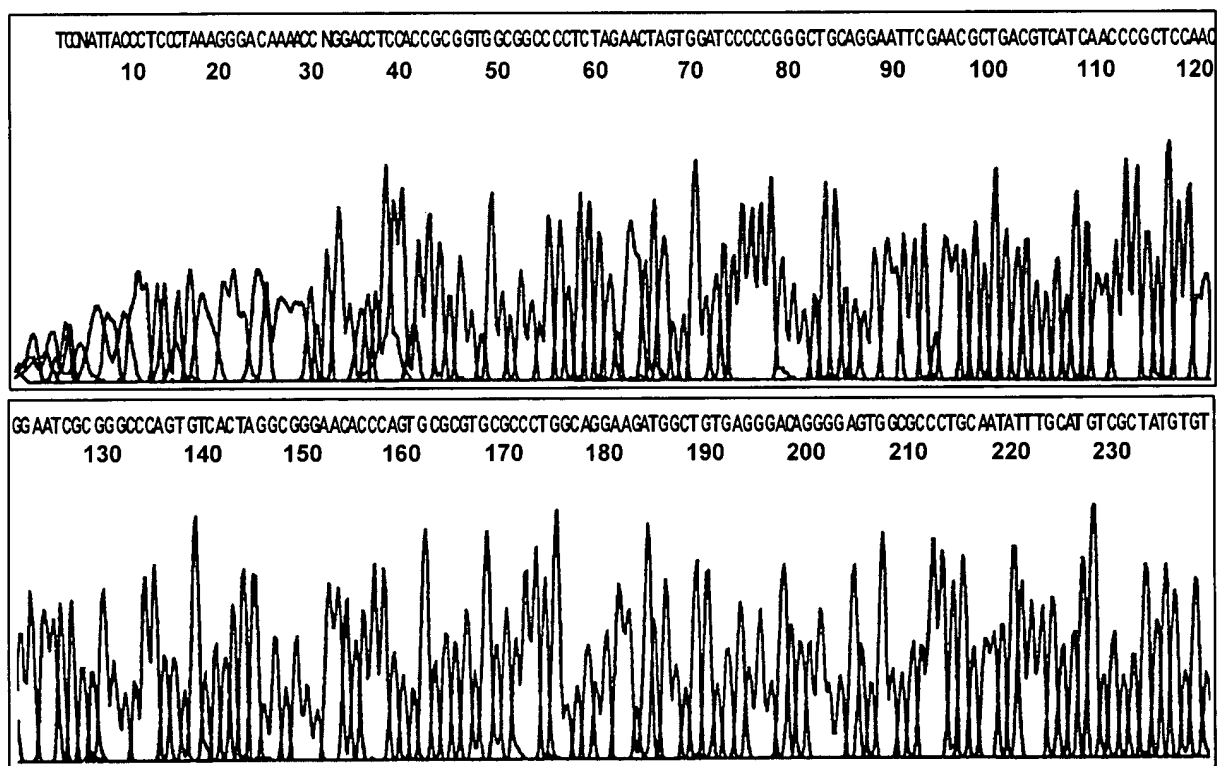
FIG. 4E-2

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FIG. 4F-1

FIG. 4F-2

FIG. 4F



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FIG. 4F-1

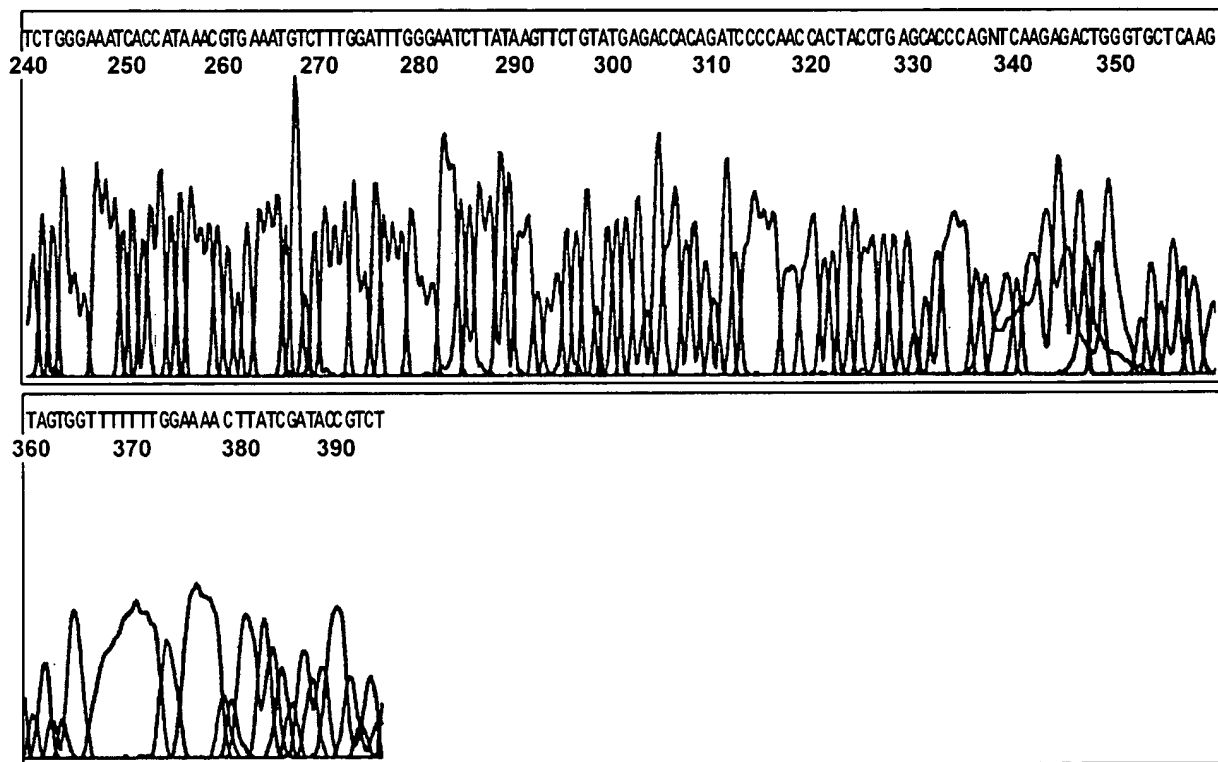


FIG. 4F-2

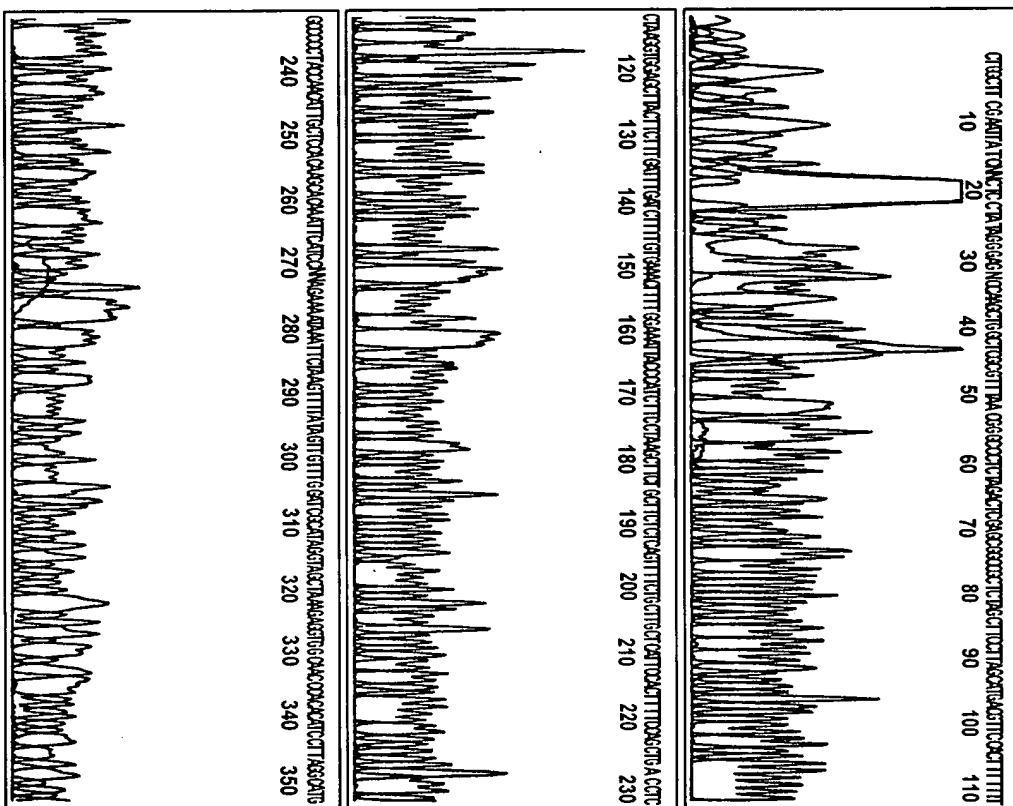


FIG. 4G

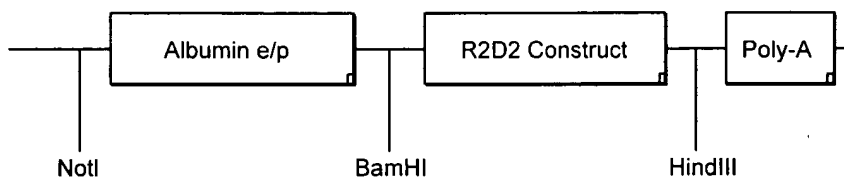


FIG. 5A

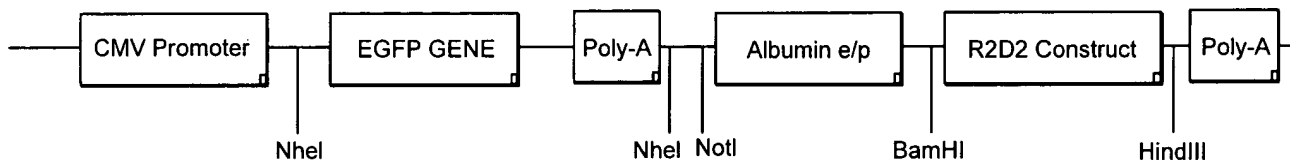


FIG. 5B

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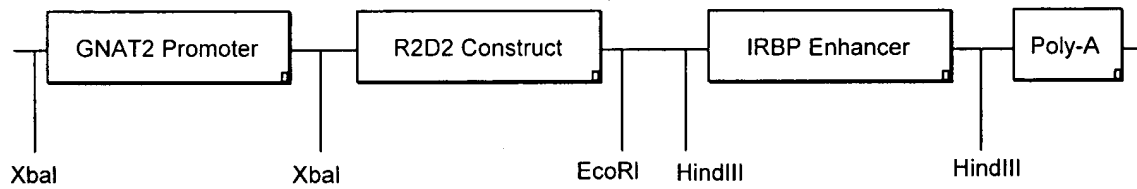
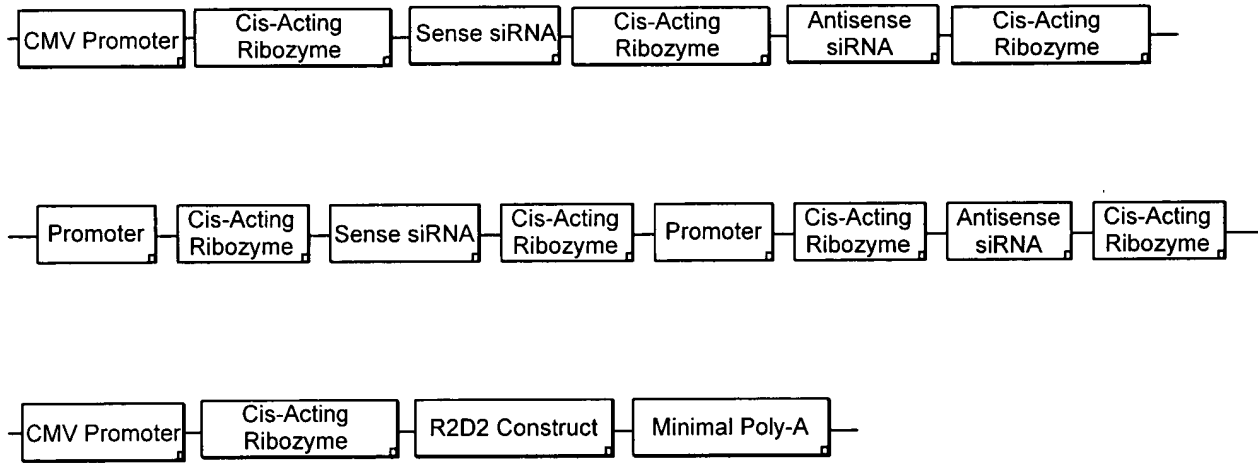


FIG. 5C



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FIG. 6

